ECONOMIC ANALYSIS IN CLINICAL RESEARCH

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OBJECTIVES:

At the completion of this lecture, the student will be able to:

1. Understand the role of economic analysis in clinical research;

2. Be familiar with economic concepts of cost and cost domains;

3. Be familiar with the different modes of economic analysis that are applied to healthcare interventions;

4. Understand the basic elements of cost-effectiveness (utility) analysis;

5. Appreciate the opportunities for, and the limitations of, conducting economic studies in conjunction with clinical trials;

6. Be familiar with potential data sources and methods of data collection for conducting economic studies in conjunction with clinical trials.

OUTLINE:

I. Introduction

In the last decade cost containment has become a major policy issue in regard to healthcare delivery in the United States. The growing prominence of economic considerations in healthcare has been reflected by an increase in the volume of literature on the economic analysis of healthcare interventions. Between 1966 and 1990 this literature grew at a fairly constant exponential rate, from an average of five studies per year in 1966 to 250 in 1990. (Elixhauser 1993) Studies involving the inclusion of economic endpoints in clinical research studies have also grown, although not necessarily as a proportion of all clinical trials conducted. (Adams 1992) However, there has been an increasing appreciation of the potential importance, as well as the limitations, of economic data from clinical trials. (McCabe 1995)

Along with a growing interest in the economic aspects of healthcare delivery and an expansion in the literature on the economic analysis of clinical studies, there has been a growing concern that much of the published literature in this area fails to meet minimum standards of methodological rigor for economic analysis. (Udvarhelyi 1992) As a result a number of guidelines or recommended standards and practices on how to conduct and read economic studies of health

care interventions have recently been promulgated by clinical journals, professional societies and government agencies; for example, Drummond, et al. (1996) provides such guidelines for economic submissions to the British Medical Journal and cites half-a-dozen other guidelines from other journals, associations and government agencies. (For a dissenting view on the usefulness of guidelines, see Evans 1995). Recently an extensive guide to methodological considerations in conducting cost-effectiveness studies related to health care interventions was published as the result of an expert panel sponsored by the U.S. Department of Health and Human Services. (Gold 1996)

Given the increasing prominence of economic considerations in clinical research it likely that a clinical researcher operating in today's environment may be asked to become involved in collecting economic data as an aspect of clinical research, and it is certainly likely that today's clinical researcher will be called upon to evaluate the results of economic research as a reader of such material in clinical or health services research journals (Drummond 1997), or as a journal referees or peer reviewer of research applications. Therefore, a knowledge of basic economic concepts and how these concepts are properly applied in the collection and evaluation of economic data should be useful to health professionals involved in the conduct of research.

II. What is the purpose of economic analysis in health care research?

A. As a guide to decision makers for making health care resource allocation decisions.

1. Health care policy, planning and program officials

2. Health care consumers and providers

B. As a tool of investigation and analysis for understanding organizational and technical factors which contribute to improved health care delivery.

C. As a tool for understanding factors which determine the distribution of health care resources, patterns of care and access to care.

III. What is cost?

A. Cost is the use of productive economic resources.

1. Resource units

2. Unit costs

B. Cost is foregone economic opportunity.

C. Transfer costs and the perspective of analysis.

D. Incremental costs (the extensive margin).

E. Marginal costs (the intensive margin).

1. The Ramsey principle vs. Reinhardt's dehospitalization mania (Reinhardt 1995)

2. Short-run vs Long-run marginal cost and the paradox of excess capital (Gold 1996)

IV. Domains of cost (Brown 1996)

A. Direct costs

1. Health care costs

2. Non-health care costs

B. Market and non-market costs

1. Unpaid labor

2. Time costs

C. Productivity costs

1. Morbidity costs

2. Mortality costs

3. Frictional costs

V. Cost and time

A. Adjusting for inflation (Sensenig 1996)

B. Discounting future costs

VI. Modes of economic analysis

A. Cost-of-Illness Studies

B. Cost-Impact Analysis

C. Healthcare Supply-side Studies

D. Access to Care Studies

E. Cost-Benefit Analysis

D. Cost-Effectiveness Analysis

E. Cost-Utility Analysis

F. Cost-Minimization Analysis

G. Cost-Savings Analysis

H. Operations Research Analysis

I. Industrial-Organization Studies

VII. General considerations in cost-effectiveness (utility) analysis (Gold 1996)

A. Perspective

1. The social perspective
2. Other perspectives

B. Alternative (comparison) program

1. Next best alternative
2. Community practice
3. Doing nothing
4. Programs of varying intensity or duration

C. Boundaries of analysis

D. Time horizon

E. The role of randomized clinical trials

1. Source of inputs into distinct cost-effectiveness analysis
2. “Piggyback” economics studies added onto RCT
3. Cost-effectiveness trial

F. The role of mathematical models in cost-effectiveness analysis

1. A framework for integrating clinical and economic data
2. A method for extrapolating results from RCTs
    a. Beyond time-frame of RCT
    b. To broader/different target population
    c. To different dose/intensity/duration

G. Dealing with uncertainty in cost-effectiveness models

1. Complexity of cost-effectiveness estimates
    a. Multiple types of data
    b. Ratio estimate of absolute differences
    c. Data from stochastic and non-stochastic sources
2. Methods for expressing uncertainty
    a. Sensitivity analysis
    b. Statistical techniques
    c. Model uncertainty

VIII. General consideration in collecting economic data in the context of a clinical trial (Drummond 1996)

A. Is the economic component of the trial important and clearly defined?

B. What perspective is supported by the data collected in the trial?

C. What are the alternative programs being evaluated in the trial and are they relevant?

D. What form of economic analysis is the trial designed to support?

E. Effectiveness data: single trial vs systematic review or meta-analysis.

F. Health outcome measures

1. What is nature of the health outcome measure (e.g., cases detected, morbidity avoided, life-years, quality-adjusted life-years)?

2. How are health benefits valued (e.g., to obtained quality-of-life measures or quality-adjusted life-years)?

G. Economic outcome measures

1. What domains of cost are covered?
2. Cost accounting

    a. Macro-costing vs micro-costing

    b. Accounting for resource units and unit costs separately

H. External validity

1. Is the technology and organization of the innovation mature enough so that costs as measured in the trial will be relevant once technology is disseminated in community practice?

2. Are the selection criteria for the trial broad enough so that the economic (as well as health) outcomes observed can be applied (or extrapolated) to the community setting?

3. Have costs associated purely with the research aspects of the trial (and not directly associated with the relevant health outcomes of the trial) been excluded from the computation of cost?

IX. Potential sources of economic data and limitations

A. Potential sources of data

1. Clinical data forms/medical records abstraction

2. Hospital bills

3. Health system cost-accounting systems (e.g. HMOs) (Nefcy 1992, Taplin 1995, Barnett 1997)

4. Administrative claims data (e.g. Medicare) (Potosky 1993, Riley 1995)

5. Patient/provider survey (Edwards 1989, Bennett 1991)

6. Cost scenario (Sofaer 1990)

B. Limitations

1. Clinical data forms

   a. Only resource units

   b. Only selected items

2. Hospital bills

   a. Need to adjust charges to costs (Finkler 1982, Shwartz 1995)

   b. May not be representative

   c. May differ from institution to institution

   d. Do not capture outpatient services

3. Health system cost-accounting systems

   a. Only includes covered services

   b. Accounting costs may not equal economic costs

   c. May not be representative

4. Administrative claims data

    a. Only covers selected populations (e.g. >64)

    b. Must be linked to clinical trial

    c. Only includes covered services

5. Patient/provider survey

    a. May be very expensive

    b. May be burdensome on respondent

    c. Respondent recall may be incomplete or biased

6. Cost scenario

    a. Depends on comprehensiveness and reliability of expert opinion

    b. May depend heavily on secondary data

    c. May be expensive to validate

X. Statistical Issues

A. Economic data are complex and multi-dimensional.

B. Economic data may be highly skewed, censored and not be easily transformable to a normal distribution. (Van Rutten 1994)

C. Standard survival methods may not be directly applicable to economic data, but alternative appropriate methods are being developed. (Etzioni 1997)

D. Trials designed for clinical outcomes may be under-powered with regard to economic endpoints.

XI. Illustrative Examples

RECOMMENDED READING:

Drummond MF, Richardson WS, O'Brien B, Levine M, Heyland D (for the Evidence-Based Medicine Working Group). 1997. Users's guides to the medical literature: XIII. How to use an article on economic analysis of clinical practice. Journal of the American Medical Association 277: 1552-1557.

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Riley GF, Potosky AL, Lubitz JD, Kessler LG. 1995. Medicare Payments from Diagnosis to Death for Elderly Cancer Patients by Stage at Diagnosis. *Medical Care* 33:828-841.

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Shwartz M, Young DW, Siegrist R. 1995. The ratio of costs to charges: how good a basis for estimating costs? Inquiry 32:476-481.

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